January 20, 2025

Subject: Letter of support for **TRAN1-16907**

Hematopoietic Stem Cell Gene Therapy for MPSIIIB (Sanfilippo B) Syndrome

Dear Members of the Application Review Subcommittee:

My name is Sophia Dai. I am a long time California resident and I currently work as a program manager at a biotechnology company focused on cell therapies in oncology. I am also a friend of a California family affected by MPSIIIB (Sanfillippo Syndrome Type B). I respectfully urge you to support the funding for **TRAN1-16907, Hematopoietic Stem Cell Gene Therapy for MPSIIIB (Sanfilippo B) Syndrome** led by principal investigator **Katelyn Masiuk**, MD, PhD.

This past Thanksgiving was a joyous time with family, including 3-year old Matthew who was recently diagnosed with MPSIIIB. I watched my nephew (also age 3) chase around his favorite cousin Matthew and give each other big bear hugs. Matthew is always sweet, caring and gentle with his cousin. It is heartbreaking to think that in the coming years, this neurodegenerative disease with a 100% mortality rate will take away all the skills Matthew has gained – physically and mentally.

The Grants Working Group (GWG) was moved by the positive results in a Sanfilippo B mouse model and curative results seen in Sanfilippo A children using the same lentiviral stem cell approach proposed by Dr. Masiuk for this study. We are hoping that the CIRM can fund this research to get us closer to a cure for children like Matthew.

As someone who has worked for the last seven years in the cell and gene therapy space, I understand the challenges of bringing therapies to fruition. Research for rare diseases often struggles to attract sufficient funding due to the limited patient population. This is why support from the CIRM is crucial—it bridges the gap and allows promising therapies to move forward when they might otherwise stall.

For Matthew and countless other children fighting against time, this funding is more than a scientific endeavor—it's a lifeline. On behalf of our family and the rare disease community, I implore you to prioritize the proposed research for Sanfilippo Syndrome B. Your support could change the trajectory of this disease and provide hope to families who need it most.

Thank you for considering this vital request.

Sincerely,

Sophia Dai